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(54) Title: ENHANCED PRODUCTION OF FUNCTIONAL PROTEINS FROM DEFECTIVE GENES

(57) Abstract: This invention provides methods for enhancing production in a subject of a functional protein from a gene disrupted by a mutation, for example by the presence of a premature stop codon or by an exon skipping mutation, comprising administering to the subject an amount of an agent effective to suppress the mutation and an amount of an agent effective to increase transcription of the gene.



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